

IMACS FORM 00: CLINICAL TRIAL DESIGN FEATURES

To be completed for all trials in the registry

GENERAL INFORMATION

Name/number of trial: _____

Principal investigator (name, affiliation, email, phone): _____

Agent(s) under investigation: _____

Phase of trial (check all that apply):

Phase 1

Phase 2

Phase 3

Phase 4

Other: _____

Number of subjects enrolled in the trial _____

Number of subjects who met primary improvement criteria _____

Number of subjects withdrawn from the trial during treatment phase _____

Number of sites which enrolled subjects _____

Date enrollment started for this study/trial: (mm/dd/yyyy) ____/____/____

Date enrollment concluded for this study/trial: (mm/dd/yyyy) ____/____/____

INCLUSION/EXCLUSION CRITERIA FOR TRIAL ENTRY:

Myositis Primary Clinical Groups included in trial: (check all that apply):

☐ Adult OR ☐ Juvenile

☐ Polymyositis

☐ Dermatomyositis

☐ Inclusion body myositis

☐ Other: please clarify _____

Classification Criteria used for Trial entry: (check all that apply):

____ Bohan and Peter criteria for IIM

____ Griggs criteria for IBM

____ Other classification criteria used: Specify _____

Was a muscle biopsy at baseline required for trial entry?

Yes

No

Inclusion Criteria for Trial Entry (check all that apply):

- ☐ Muscle strength less than a certain strength: _____
- ☐ Disease activity > certain amount: _____
- ☐ Specified level of functional disability: _____
- ☐ Refractory disease with inadequate response to first- line agents such as corticosteroids and methotrexate
- ☐ New onset disease: _____
- ☐ Inadequate response to other therapeutic agents _____
- ☐ Unacceptable corticosteroid toxicity _____
- ☐ Cutaneous or other extra-muscular manifestations: _____

Definition of Inadequate Response to First Line Agents: (check all that apply):

- ☐ Adequate corticosteroid treatment trial to define treatment failure was agreed to be 60 mg/day for at least 2 months in adult patients, and 2.0 mg/kg/day prednisone for at least 2.5 months in pediatric patients
- ☐ Methotrexate treatment failure in pediatric patients was agreed to be 25 mg/m²/week parenterally for at least 3 months duration.
- ☐ Other definitions used: _____

Exclusion criteria for trial entry: (check all that apply):

- ☐ Myositis associated with malignancy
- ☐ Myositis associated with another connective tissue disease
- ☐ Myositis associated with an environmental risk factor (penicillamine, collagen implants, etc.)
- ☐ Significant organ system involvement: _____
- ☐ Significant myositis damage _____
- ☐ Hepatic disease
- ☐ Other _____

Allowable Concomitant Therapy: (complete all that apply):

- ☐ Prednisone: Dose _____
- ☐ Methotrexate: dose _____
- ☐ Other medications- list and dose _____
- ☐ Physical therapy- continued, stable regimen
- ☐ Other: _____

Was a standard dose reduction regimen used for corticosteroid tapering? ☐ Yes ☐ No
If so, please include: _____

Trial Design:

- Double-blinded
- Placebo controlled: Duration placebo phase: _____
- Cross over
- Direct comparison to active agent
- Open label
- Other: _____

Trial Duration:

____ Months for active treatment phase

____ Months for open-label follow-up after active treatment phase

Assessment Intervals for Efficacy and Safety:

Every ____ months during active treatment phase

Every ____ months during open label follow-up phase after completion of active treatment

Safety Assessment:

____ NCI Common Toxicity Criteria

____ Other _____

Trial outcome measures (check all that apply and specify primary or secondary endpoint):

____ IMACS Preliminary Definitions of Improvement _____

____ IMACS Core set activity measures _____

____ PRINTO Preliminary definitions of Improvement _____

____ PRINTO Core set activity measures _____

____ Corticosteroid dose reduction

____ Time to complete clinical response

____ Other _____

Trial dropout criteria: (check all that apply):

____ Physician global worsening of ≥ 2 cm on a 10cm VAS and a worsening of the manual muscle testing by $\geq 20\%$, or

____ Extramuscular organ disease activity worsening by ≥ 2 cm on a 10cm VAS,

____ Any 3 of 6 IMACS core set activity measures worse by $\geq 30\%$

____ Other _____

Trial Flare Criteria: did your trial use a definition of flare? ____ Yes ____ No

If yes, as a trial endpoint? ____ As withdrawal criteria? ____

If yes, specify definition of flare used _____

If yes, % of subjects who met flare criteria in the study _____

COMPLETE CLINICAL RESPONSE/REMISSION:

Complete clinical response:

Was complete clinical response assessed in the trial? ____ Yes ____ No (if no skip to Remission)

If yes, did your trial use IMACS complete clinical response criteria (6-month continuous period of no evidence of disease activity while still on myositis therapy) as a trial endpoint? ____ Yes ____ No

Did you use a different definition than the one specified above? If yes, please specify:

What % of subjects vs. controls achieved a complete clinical response in your trial?

Patients: _____ % Controls: _____ %

What was the mean duration and range of complete clinical response (in months) in your trial?

Mean duration complete clinical response: _____ months

Minimum duration complete clinical response: _____ months

Maximum duration complete clinical response _____ months

Remission:

Was remission assessed in the trial? ____ Yes ____ No (if no skip to Analyses)

Did your trial use IMACS remission criteria (6-month continuous period of no evidence of disease activity while off myositis therapy) as a trial endpoint? ____ Yes ____ No

Did you use a different definition of remission than the one specified above? If yes, please specify: _____

What % of patients vs. controls achieved remission in your trial?

Patients: _____ % Controls: _____ %

What was the mean duration and range of remission (in months) in your trial?

Mean duration remission: _____ months

Minimum duration remission: _____ months

Maximum duration remission: _____ months

TRIAL ANALYSES:

Primary outcome analyses performed: (check all that apply)

____ Intention to treat

____ Last observation carried forward

____ Other analyses performed

Post-hoc stratification:

Did you perform any post-hoc stratification? ____ Yes ____ No

If yes, please specify the post-hoc stratification variables assessed: (check all that apply)

____ Clinical group,

____ Duration of disease,

____ Degree of muscle weakness/dysfunction at enrollment,
____ Extramuscular organ involvement: _____
____ Autoantibodies: _____
____ Muscle histopathology: _____
____ Cutaneous or gastrointestinal ulceration
____ Calcinosis
____ Other: _____